

## Clinical Policy: Cysteamine ophthalmic (Cystaran, Cystadrops)

Reference Number: PA.CP.PMN.130

Effective Date: 04/2019

Last Review Date: 04/2024

### Description

Cysteamine (Cystaran<sup>®</sup>, Cystadrops<sup>®</sup>) ophthalmic solution is a cystine-depleting agent.

### FDA Approved Indication(s)

Cystaran and Cystadrops are indicated for the treatment of corneal cystine crystal accumulation in patients with cystinosis.

### Policy/Criteria

*Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.*

It is the policy of PA Health & Wellness<sup>®</sup> that Cystaran and Cystadrops are **medically necessary** when the following criteria are met:

### I. Initial Approval Criteria

#### A. Corneal Cystine Crystal Accumulation (must meet all):

1. Diagnosis of cystinosis;
2. Prescribed by or in consultation with an ophthalmologist;
3. Presence of corneal cystine accumulation;
4. Dose does not exceed one of the following (a or b):
  - a. Cystaran: 1 drop in each eye every hour while awake (1 bottle per week);
  - b. Cystadrops: 1 drop in each eye 4 times a day while awake (1 bottle per week).

**Approval duration: 6 months**

#### B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53.

### II. Continued Therapy

#### A. Corneal Cystine Crystal Accumulation (must meet all):

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.LTSS.PHAR.01) applies;
2. Member is responding positively to therapy;
3. If request is for a dose increase, new dose does not exceed one of the following (a or b):
  - a. Cystaran: 1 drop in each eye every hour while awake (1 bottle per week);
  - b. Cystadrops: 1 drop in each eye 4 times a day while awake (1 bottle per week).

**Approval duration: 12 months**

**B. Other diagnoses/indications (must meet 1 or 2):**

1. Currently receiving medication via PA Health & Wellness benefit or member has previously met all initial approval criteria or the Continuity of Care Policy (PA.LTSS.PHAR.01) applies.

**Approval duration: Duration of request or 12 months (whichever is less);** or

2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized PA.CP.PMN.53.

**III. Diagnoses/Indications for which coverage is NOT authorized:**

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policy – PA.CP.PMN.53 or evidence of coverage documents.

**IV. Appendices/General Information**

*Appendix A: Abbreviation/Acronym Key*

FDA: Food and Drug Administration

*Appendix B: Therapeutic Alternatives*

Not applicable

*Appendix C: Contraindications/Boxed Warnings*

None reported

**V. Dosage and Administration**

Indication	Dosing Regimen	Maximum Dose
Cystaran (cysteamine)	1 drop in each eye every waking hour	1 drop/eye/hour during waking hours
Cystadrops (cysteamine)	1 drop in each eye, 4 times a day during waking hours	See dosing regimen

**VI. Product Availability**

Drug Name	Availability
Cystaran (cysteamine)	Ophthalmic solution: 6.5 mg/mL of cysteamine hydrochloride equivalent to 4.4 mg/mL of cysteamine (0.44%)
Cystadrops (cysteamine)	Ophthalmic solution containing 3.8 mg/mL of cysteamine (0.37%) in 5 mL bottle

**VII. References**

1. Cystaran Prescribing Information. Gaithersburg, MD: Leadiant Biosciences, Inc., May 2023. Available at: <http://www.cystaran.com/>. Accessed January 12, 2024.
2. Cystadrops Prescribing Information. Lebanon, NJ: Recordati Rare Diseases Inc.; August 2020. Available at: <https://www.cystadrops.com>. Accessed February 1, 2024.
3. Cystinosis. National Organization for Rare Disorders website. <https://rarediseases.org/rare-diseases/cystinosis/>. Published 1986. Updated 2020. Accessed February 1, 2024

## CLINICAL POLICY

### Cysteamine ophthalmic



4. Elmonem MA, Veys KR, Soliman NA, et. al. Cystinosis: a review. *Orphanet J Rare Dis*. 2016 Apr 22; 11: 47.

Reviews, Revisions, and Approvals	Date
Policy created.	04/2019
2Q 2020 annual review: added appendix C; references reviewed and updated	04/2020
2Q 2021 annual review: added Cystadrops to policy; references reviewed and updated.	04/2021
2Q 2022 annual review: references reviewed and updated.	04/2022
2Q 2023 annual review: no significant changes; references reviewed and updated.	04/2023
2Q 2024 annual review: no significant changes; references reviewed and updated.	04/2024